

Placental Stem Cells for the In Utero Treatment of Spina Bifida

Grant Award Details

Placental Stem Cells for the In Utero Treatment of Spina Bifida

Grant Type: Preclinical Development Awards

Grant Number: PC1-08103

Project Objective: Development of placental stem cells as a candidate for the in utero treatment of spina bifida.

Investigator:

Name: Diana Farmer

Institution: University of California, Davis

Type: PI

Name: Aijun Wang

Institution: University of California, Davis

Type: Co-PI

Disease Focus: Neurological Disorders, Pediatrics, Spina Bifida

Human Stem Cell Use: Adult Stem Cell

Cell Line Generation: Adult Stem Cell

Award Value: \$2,182,146

Status: Active

Progress Reports

Reporting Period: Year 1

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Grant Application Details

Application Title: Placental Stem Cells for the In Utero Treatment of Spina Bifida

Public Abstract:

Myelomeningocele – also known as spina bifida – is a devastating and costly defect that causes lifelong paralysis as well as bowel and bladder incontinence in newborns. It is one of the most common birth defects worldwide, with four children in the United States born with spina bifida every day. Spina bifida affects the physical, educational, social, and psychological development of these children. Most patients require multiple surgeries and hospitalizations throughout their lives. Physicians are now able to diagnose this disease during pregnancy, and new fetal surgical techniques allow surgeons to safely operate on these children in the womb. This unique fetal surgery was studied in the award winning Management of Myelomeningocele Study (MOMS). The MOMS trial showed – for the first time ever – that the paralysis associated with spina bifida could be improved. Children treated in the womb were more likely to walk independently than those who were repaired after birth. However, the improvements seen were not perfect and the majority of children treated with fetal surgery still had some level of paralysis or lower extremity weakness.

Our research has built upon the success of the MOMS trial to address the residual deficits seen in children even after treatment with fetal surgery. We have developed a placental stem cell based therapy that can be applied at the time of fetal repair, in order to reverse spinal cord damage. After six years of laboratory research investigating different stem cell types and the best way to deliver a stem cell based treatment in the womb, we have discovered a placental stem cell therapy that cures spina bifida in the animal model. Animals treated with these cells can make a full recovery and are able to walk normally without any evidence of lower extremity paralysis. These amazing results require additional testing and FDA approval before the therapy can be used in humans. With this proposal, we will optimize this stem cell product, validate its effectiveness, determine the optimal dose, and confirm its preliminary safety in order to translate this new treatment to clinical trials. Stem cell therapy for spina bifida could cure this devastating disease, alleviating a massive burden on children, families, and society.

Statement of Benefit to California:

Spina bifida is one of the most common, costly, and disabling birth defects. Within the United States, four children per day are born with this devastating disease. In California, the 5-year statewide incidence of spina bifida was 6.8 cases per 10,000 live births between 1999 and 2003, significantly higher than the Healthy People 2010 target of 3 per 10,000 births. Additionally, spina bifida disproportionately affects Americans of Hispanic and Latino descent, who make up 37.6% of California's population. Given the disproportionately high incidence of children born with spina bifida in California, and the lifelong disability these children live with, spina bifida is a substantial economic burden to the state. The estimated average total lifetime cost to California is approximately \$532,000 for each child born with spina bifida. However, for many children, the cost may be several million dollars due to repeat surgical procedures, frequent hospitalizations, and the need for ongoing physical and cognitive rehabilitation. In addition to the direct medical costs associated with spina bifida, the indirect costs include: pain and suffering, cost of specialized childcare, and the lost earning potential of unpaid caregivers, which compound the impact the disease has on California's economy.

There is currently no cure for spina bifida, and interventions that mitigate the negative consequences of the disease (lower body paralysis, bowel and bladder incontinence) are urgently needed. For the first time, hope for an improved treatment option was provided by the award winning Management of Myelomeningocele Study (MOMS). The MOMS trial was a multicenter randomized controlled trial demonstrating that the paralysis associated with spina bifida might be improved by surgical repair of the defect before birth. This promise of fetal intervention for spina bifida was based on the hypothesis that early in utero treatment would have the potential to fix the defect before permanent spinal cord damage occurred. While the MOMS trial did demonstrate an improvement in the lower extremity paralysis of those patients undergoing in utero repair compared to postnatal repair, these improvements were not universal for all children. This proposal presents an innovative placental stem cell-based therapy to augment fetal repair and further improve and possibly cure the devastating and costly neurologic deficits of spina bifida. A cure for spina bifida would relieve California families and society of the tremendous emotional and economic cost burden of this debilitating disease, and would be life changing for future children afflicted with spina bifida.

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